CIRM Board Approves Four New Clinical Trials

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OAKLAND, CA – Today the governing Board of the California Institute for Regenerative Medicine (CIRM) approved four new clinical trials in addition to ten new discovery research awards.

These new awards bring the total number of CIRM-funded clinical trials to 68. Additionally, these new additions have allowed the state agency to exceed the goal of commencing 50 new trials outlined in its five year strategic plan.

$8,970,732 was awarded to Dr. Steven Deeks at the University of California San Francisco (UCSF) to conduct a clinical trial that modifies a patient's own immune cells in order to treat and potentially cure HIV.

Current treatment of HIV involves the use of long-term antiretroviral therapy (ART). However, many people are not able to access and adhere to long-term ART.

Dr. Deeks and his team will take a patient's blood and extract T cells, a type of immune cell. The T cells are then genetically modified to express two different chimeric antigen receptors (CAR), which enable the newly created duoCAR-T cells to recognize and destroy HIV infected cells. The modified T cells are then reintroduced back into the patient.

The goal of this one time therapy is to act as a long-term control of HIV with patients no longer needing to take ART, in effect a form of HIV cure. This approach would also address the needs of those who are not able to respond to current approaches, which is estimated to be 50% of those affected by HIV globally.

$3,728,485 was awarded to Dr. Gayatri Rao from Rocket Pharmaceuticals to conduct a clinical trial using a gene therapy for infantile malignant osteopetrosis (IMO), a rare and life-threatening disorder that develops in infancy. IMO is caused by defective bone cell function, which results in blindness, deafness, bone marrow failure, and death very early in life.

The trial will use a gene therapy that targets IMO caused by mutations in the TCIRG1 gene. The team will take a young child's own blood stem cells and inserting a functional version of the TCIRG1 gene. The newly corrected blood stem cells are then introduced back into the child, with the hope of halting or preventing the progression of IMO in young children before much damage can occur.

Rocket Pharmaceuticals has used the same gene therapy approach for modifying blood stem cells in a separate CIRM funded trial for a rare pediatric disease, which has shown promising results.

$8,996,474 was awarded to Dr. Diana Farmer at UC Davis to conduct a clinical trial of *in utero* repair of myelomeningocele (MMC), the most severe form of spina bifida. MMC is a birth defect that occurs due to incomplete closure of the developing spinal cord, resulting in neurological damage to the exposed cord. This damage leads to lifelong lower body paralysis, and bladder and bowel dysfunction.

Dr. Farmer and her team will use placenta tissue to generate mesenchymal stem cells (MSCs). The newly generated MSCs will be seeded onto an FDA approved dural graft and the product will be applied to the spinal cord while the infant is still developing in the womb. The goal of this therapy is to help promote proper spinal cord formation and improve motor function, bladder function, and bowel function.

The clinical trial builds upon the work of CIRM funded preclinical research.

$8,333,581 was awarded to Dr. David Williams at Boston Children's Hospital to conduct a gene therapy clinical trial for sickle cell disease (SCD). This is the second project that is part of an agreement between CIRM and the National Heart, Lung, and Blood Institute (NHLBI), part of the National Institutes of Health, to co-fund cell and gene therapy programs under the NHLBI's "Cure Sickle Cell" Initiative. The goal of this agreement is to markedly accelerate clinical development of cell and gene therapies to cure SCD.

SCD is an inherited disease caused by a single gene mutation resulting in abnormal hemoglobin, which causes red blood cells to 'sickle' in shape. Sickling of red blood cells clogs blood vessels and leads to progressive organ damage, pain crises, reduced quality of life, and

early death.

The team will take a patient's own blood stem cells and insert a novel engineered gene to silence abnormal hemoglobin and induce normal fetal hemoglobin expression. The modified blood stem cells will then be reintroduced back into the patient. The goal of this therapy is to aid in the production of normal shaped red blood cells, thereby reducing the severity of the disease.

"Today is a momentous occasion as CIRM reaches 51 new clinical trials, surpassing one of the goals outlined in its five year strategic plan," says Maria T. Millan, M.D., President and CEO of CIRM. "These four new trials, which implement innovative approaches in the field of regenerative medicine, reflect CIRM's ever expanding and diverse clinical portfolio."

The Board also approved ten awards that are part of CIRM's Quest Awards Program (DISC2), which promote promising new technologies that could be translated to enable broad use and improve patient care.

The awards are summarized in the table below:

APPLICATION	TITLE	INSTITUTION	AWARD AMOUNT
DISC2-12169	Human-induced pluripotent stem cell-derived glial enriched progenitors to treat white matter stroke and vascular dementia.	UCLA	$250,000
DISC2-12170	Development of COVID-19 Antiviral Therapy Using Human iPSC-Derived Lung Organoids	UC San Diego	$250,000
DISC2-12111	Hematopoietic Stem Cell Gene Therapy for X-linked Agammaglobulinemia	UCLA	$250,000
DISC2-12158	Development of a SYF2 antisense oligonucleotide (ASO) treatment for ALS	University of Southern California	$249,997
DISC2-12124	Dual angiogenic and immunomodulating nanotechnology for subcutaneous stem cell derived islet transplantation for the treatment of diabetes	Lundquist Institute	$250,000
DISC2-12105	Human iPSC-derived chimeric antigen receptor-expressing macrophages for cancer treatment	UC San Diego	$250,000
DISC2-12164	Optimization of a human interneuron cell therapy for traumatic brain injury	UC Irvine	$250,000
DISC2-12172	Combating COVID-19 using human PSC-derived NK cells	City of Hope	$249,998

DISC2-12126	The First Orally Delivered Cell Therapy for the Treatment of Inflammatory Bowel Disease	Vitabolus Inc.	$249,000
DISC2-12130	Transplantation of Pluripotent Stem Cell Derived Microglia for the Treatment of Adult-onset Leukoencephalopathy (HDLS/ALSP)	UC Irvine	$249,968

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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